
Human Embryonic Stem Cell-Derived Cardiomyocytes for Patients with End Stage Heart Failure

Grant Award Details

Human Embryonic Stem Cell-Derived Cardiomyocytes for Patients with End Stage Heart Failure

Grant Type: Disease Team Therapy Planning I

Grant Number: DR2-05394

Investigator:

Name:	Robert Robbins
Institution:	Stanford University
Type:	PI

Disease Focus: Heart Disease

Award Value: \$73,030

Status: Closed

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Human Embryonic Stem Cell-Derived Cardiomyocytes for Patients with End Stage Heart Failure

Public Abstract: Patients with end-stage heart failure (ESHF) have a 2-year survival rate of 50% with conventional medical therapy. This dismal survival rate is actually significantly worse than patients with AIDS, liver cirrhosis, stroke, and other debilitating diseases. Stem cell therapy may be a promising strategy for inducing myocardial regeneration via paracrine activation, prevention of cardiac apoptosis, and other mechanisms. Several studies have convincingly shown that human embryonic stem cells can be differentiated into cardiomyocytes (hESC-CMs) and that these cells can be used to effectively improve cardiac function following myocardial infarction (MI). The objectives of this CIRM Disease Team Therapy proposal are two-fold: (1) to perform IND enabling studies involving hESC-CM for subsequent FDA approval and (2) to complete a Phase I trial with ESHF patients undergoing the left ventricular assist device (LVAD) procedure whereby hESC-CMs will be injected at the same time.

**Statement of Benefit to
California:**

Coronary artery disease (CAD) is the number one cause of mortality and morbidity in the US. Following myocardial infarction (MI), the limited ability of the surviving cardiac cells to proliferate thereafter renders the damaged heart susceptible to dangerous consequences such as heart failure. In recent years, stem cell therapy has emerged as a promising candidate for treating ischemic heart disease. In contrast to adult stem cells, human embryonic stem cells (hESCs) have the advantage of being pluripotent, which endows them with the ability to differentiate into virtually every cell type. Numerous studies have demonstrated that hESC-derived cardiomyocytes (hESC-CMs) can improve cardiac function in small and large animal models. In addition, the FDA has approved hESC-derived oligodendrocyte progenitor cells for patients with acute spinal cord injury and hESC-derived retinal pigment epithelial cells for patients with Stargardt's macular dystrophy. Hence the conventional controversies and regulatory hurdles related to hESC-based trials are no longer major barriers to the field. In this proposal, we seek to extend and translate the robust pre-clinical data into clinical reality by demonstrating the safety and feasibility of hESC-CM transplantation. We will perform careful IND-enabling research in the first 3 years. Afterwards, our medical teams will initiate a phase 1 clinical trial involving 10 patients with end stage heart failure (ESHF). We will perform direct intramyocardial injection of hESC-CMs in ESHF patients undergoing left ventricular assist device (LVAD) implantation as a bridge toward orthotopic heart transplantation (OHT). After the patients have received matching donor hearts, the native recipient hearts will be explanted. This will provide us an opportunity to carefully assess the fate of these cells and to ensure safety before we can embark on a larger clinical trial in Years 5-10.

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